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## Skin-derived precursor cells for the treatment of enteric neuromuscular dysfunction

### Grant Award Details

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Skin-derived precursor cells for the treatment of enteric neuromuscular dysfunction

**Grant Type:** Tools and Technologies III

**Grant Number:** RT3-07914-A

**Project Objective:** Aim to establish analogous porcine models of intestinal denervation to accommodate cell-based therapies and to explore the regenerative potential of human SKP to treat the spectrum of GND disorders.

**Investigator:**

<b>Name:</b>	James Dunn
<b>Institution:</b>	University of California, Los Angeles
<b>Type:</b>	PI

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**Disease Focus:** Intestinal Disease, Metabolic Disorders, Neurological Disorders, Pediatrics

**Human Stem Cell Use:** Adult Stem Cell

**Award Value:** \$1,035,538

**Status:** Active

### Progress Reports

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**Reporting Period:** Year 1

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### Grant Application Details

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**Application Title:** Skin-derived precursor cells for the treatment of enteric neuromuscular dysfunction

**Public Abstract:**

The intestine performs the essential function of absorbing food and water into the body. Without a functional intestine, children and adults cannot eat normal meals, and these patients depend on intravenous nutrition to sustain life. Many of these patients do not have a neural system that coordinates the function of the intestine. These patients have a poor quality of life, and the cost of medical care is over \$200,000 per year for each patient. Stem cell therapies offer potential cures for these patients while avoiding the risks of invasive procedures and hazardous treatments. A novel approach to treat these patients is to use stem cells derived from the patient's own skin to generate the neural system. This has been shown to be feasible in small animals, and the next step hinges on the demonstration of these results in a large animal model of intestinal dysfunction. We will develop a model in large animals that can be used to test the ability of skin-derived stem cells to form the neural system. Skin-derived stem cells will be isolated from large animal models and human skin to demonstrate their potential to generate a functional neural system. These cells will be transplanted into the animal model to determine the best way for these cells to make the intestine function properly. This research will gather critical information needed to begin a clinical trial using skin-derived cells to treat intestinal dysfunction.

**Statement of Benefit to California:**

Gastrointestinal dysfunction destroys the lives of thousands of Californians. These Californians have frequent and prolonged hospitalizations and lost wages due to their chronic illness. It is estimated that the health care cost of Californians with gastrointestinal neuromuscular dysfunction is over 400 million dollars annually. Currently, most of these patients are covered by the state's insurance agency. Stem cell therapies offer potential cures for these patients and reduce this economic burden. The proposed research will obtain critical information needed to begin a clinical trial using skin-derived cells to treat patients with intestinal dysfunction. The California economy will significantly benefit from this research through the reduced costs for health care and increased quality of life of the affected Californians. Additionally, this work will add to the state's growing stem cell industry and will increase employment opportunities and revenue by the state of California. The educational benefit to Californians involved in this research project will also maintain California's position in leading the stem cell effort in the future.

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